# Hematopoetic stem cell gene therapy for the treatment of Tay-Sachs disease

## Grant Award Details

Hematopoetic stem cell gene therapy for the treatment of Tay-Sachs disease

**Grant Type:** Late Stage Preclinical Projects

**Grant Number:** CLIN1-14006

**Investigator:**

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| **Name:** | Joseph Anderson |
| **Institution:** | University of California, Davis |
| **Type:** | PI |

**Award Value:** $4,048,253

**Status:** Pre-Active

## Grant Application Details

**Application Title:** Hematopoetic stem cell gene therapy for the treatment of Tay-Sachs disease

**Public Abstract:**

**Therapeutic Candidate or Device**

Autologous hematopoietic stem cells transduced with a HexA/HexB expressing lentiviral vector

**Indication**

Tay-Sachs disease

**Therapeutic Mechanism**

The transplanted gene modified autologous hematopoietic stem cells will engraft in the bone marrow and start producing HexA/HexB expressing immune progeny. Microglia, which establish residence in the brain, will deliver functional beta-hexosaminidase enzyme to affected neurons and restore degradation of GM2-gangliosides.

**Unmet Medical Need**

Currently there is no cure for Tay-Sachs disease. Only palliative care is available. If successful, our therapeutic candidate will restore beta-hexosaminidase activity in the CNS of affected patients and halt disease progression.

**Project Objective**

To submit an IND to the FDA for a Phase I trial

**Major Proposed Activities**

- Evaluate the in vivo toxicity of HexA/HexB vector transduced cells in NRG mice including pathology, tumorigenesis, and vector copy number
- Manufacture and certify a clinical lot of HexA/HexB lentiviral vector for use in a future Phase I clinical trial
- Perform a manufacturing dry run for a mock drug product and submit an IND to the FDA for a future Phase I clinical trial

**Statement of Benefit to California:**

Currently, there is no cure or corrective therapy for Tay-Sachs disease, and supportive care can prolong the lives of patients only marginally. The successful development of this therapy will not only help patients with TS but will demonstrate the use case of this therapeutic approach for other monogenic neurodegenerative diseases.

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